Rewarding Innovation in Drug Development

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The development of innovative medications is largely a private enterprise that relies chiefly on the investment of pharmaceutical companies in research and development (R&D). This process can be described as a cycle, where the success of one drug is necessary to stimulate the development and eventual success of a subsequent therapy. Specifically, this "innovation cycle" includes 3 major stages—drug innovation, reward to society (ie, patient access), and reinvestment of drug sales into the development of new drugs. Of note, this cycle is an incremental process, whereby failure to reward the initial development of a drug in a specific therapeutic area may have a negative impact on—or entirely halt—the rate of continued innovation in that field.

A Lengthy, Costly, and Risky Process

The development of innovative new therapies is a lengthy, expensive, and risky process. The entire time from initial R&D through a drug's regulatory approval can take between 10 and 15 years,[1-3] at an estimated average cost ranging from $1.2 billion to more than $1.8 billion in the United States (including the cost of failures).[4-6] In a 2011 report by BlueCross BlueShield, the annual aggregate spending on R&D by the pharmaceutical industry was estimated to be $12.6 billion on the development of new therapies.[7]

Despite these high costs, the risk for drug failure is significant: only 1 of thousands of screened compounds may eventually become an approved medicine after years of clinical testing. Furthermore, although the high prices associated with novel therapies continue to be criticized, it is estimated that only 3 in 10 approved drugs recover their R&D costs.[8] This high risk underscores the importance of rewarding truly innovative medications to sustain ongoing drug development.

Patient Access Sustains R&D Innovation

Although all stages of the innovation cycle are critical for ongoing drug development, patient access to treatment, naturally, has the greatest societal impact. For patients, reward for innovation comes in the form of the availability of cutting-edge medicines and drugs with improved efficacy and/or safety profiles. Access to new and innovative therapies provides additional benefits to society as a whole, such as improved population health and quality of life, reduced hospitalizations, increased productivity, and an increasing number of jobs.[9]

Because many patients cannot afford the drugs that they require, they may be supported through payer reimbursement by private and public insurance plans. These payers have a business responsibility (eg, as a result of tax funding, member premiums) to patients and to manufacturers to support innovation and to make breakthrough, high-efficacy drugs available to the individuals who need them. The provision of patient access is vital in driving the demand for and utilization of new drugs, and the maintenance of sales is increasingly necessary to stimulate R&D and to sustain the pharmaceutical marketplace.[8,9]

Disparate Innovation Cycles: Cancer versus Obesity

To achieve the greatest health outcomes and to support the innovation cycle, promising new drugs must be rewarded across all therapeutic areas. A prime example of a successful innovation cycle is the R&D and subsequent utilization of oncology drugs. Recent data indicate that there are more pharmaceuticals under development for cancer than for any other therapeutic area, with cancer-targeted agents representing approximately 30% of all drugs currently in development in the United States.[1,10] Major reasons for the strong interest in these therapies include a substantial unmet medical need and the high market recognition that cancer drugs receive for their innovation.

A report from the World Health Organization (WHO)'s International Agency for Research on Cancer recently warned of an imminent "human disaster," based on the prediction that new cancer diagnoses will increase by 57% worldwide over the next 20 years, from 14 million cases annually in 2012 to 22 million cases annually in 2032.[11] As a result, there is likely an enhanced willingness to approve and to reimburse oncology drugs, which continue to be rewarded by private and public payers, even in countries that use cost-effectiveness as a key criterion for clinical and policy decision-making.

Truly innovative therapies for cancer are also frequently approved in a short time frame through 1 of 4 expedited review and approval processes offered by the US Food and Drug Administration. These processes include fast tracking, breakthrough status, priority review, and accelerated approval.[12] Taken collectively with disease burden, the incentives to reinvest in the development of innovative cancer treatments are significant.

Conversely, despite the epidemic status of obesity and

its associated high risks for negative clinical and economic outcomes, the absence of innovative drug therapies targeting the condition is notable. According to the WHO, 11% of adults aged ≥20 years were obese in 2008, amounting to more than 200 million men and nearly 300 million women worldwide.[13] Obesity is linked to many deleterious health effects, such as cardiovascular disease, a variety of musculoskeletal diseases, diabetes, and cancer.[13] Moreover, obese individuals are at an elevated risk for early mortality: a recent meta-analysis reported an 18% increased risk for death in morbidly obese individuals compared with the general population.[14]

Not surprising, the management of obesity and its associated health consequences places an enormous financial burden on society, with annual medical costs reaching an estimated $190 billion in the United States in 2012.[15]

Despite its high prevalence and deleterious clinical and economic consequences, successful innovation in the treatment of obesity remains rare.[16] According to an April 2014 personal e-mail communication from Evaluate Pharma, recent R&D data from its database indicate that only 190 drugs are currently under development to target obesity; in contrast, a 2013 report on drug development that was also using data from the EvaluatePharma database noted that 3436 drugs were undergoing evaluation for the treatment of cancer.[1]

This considerable disparity may relate to the common perception that obesity is a lifestyle condition that could and should be modified via diet and exercise alone.[17] Furthermore, the unmet need associated with conditions such as obesity is often regarded as less severe and may be less well understood than that of cancer, making the measurement of a therapeutic value difficult.[16,18] These factors may create challenges in terms of reimbursement, because payers may be reluctant to reimburse for medicines that treat obesity as a result of the previously mentioned perceptions of the disease.

Without the certainty of patient access and adequate return on investment, manufacturers may regard the risk associated with the development of anti-obesity drugs as too high. As such, the incremental nature of the innovation cycle becomes halted in the weight-loss pharmaceutical industry. This is a devastating situation for patients who experience significant morbidity, financial burden, and early mortality related to obesity.

Rewarding Innovation Improves Health Outcomes

Given the financial pressures to maintain government and private budgets, cost-effectiveness research is increasingly being used to inform decision-making regarding the reimbursement of new pharmaceuticals. Although such research activities certainly support the allocation of scarce healthcare resources, they are also known to limit access to innovative medicines, including those targeting cancer and obesity.[10,19,20]

In addition, the use of price controls such as cost-effectiveness reduces patient access to medications, which in turn reduces utilization and revenue, thereby taking money out of R&D initiatives within the innovation cycle. It has been suggested that if the United States adopted price controls similar to those implemented in the rest of the world (eg, the United Kingdom, Canada, Australia), manufacturers would substantially reduce drug research, and the flow of new medicines would decrease by approximately 75% over the long-term.[21]

Drug pricing and reimbursement mechanisms must therefore adequately reward innovation for medications in all therapeutic areas to speed patient access, increase global health, and strengthen the incentives for the pharmaceutical industry to further invest in R&D. This will ensure that innovation will continue to advance to further improve the health outcomes of society as a whole. ■

Author Disclosure Statement

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